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Director

Memorandum

To: **Senate Judiciary I Subcommittee on Pharmaceutical Liability**
From: **Bill Patterson, Staff Attorney**
Date: **April 24, 2012**
Re: **FDA Review Process for Drugs Intended to Treat Life-Threatening or Severely-Debilitating Illnesses**

At the subcommittee's meeting on March 29, 2012, staff was asked to determine whether the FDA employs a different standard in approving the sale of a drug that is intended to save lives. As explained below, the FDA review process employs special procedures in reviewing applications for the sale of drugs that are intended to treat life-threatening or severely-debilitating illnesses.

As part of its review of an application to market a new drug, the FDA assesses whether the health benefits of the drug outweigh the known risks for its approved use, and will not approve sale of the drug unless its analysis "establishes that a drug's benefits outweigh its known risks for its proposed use."[1]

When the application is for the sale of a drug that is intended to treat life-threatening or severely-debilitating illnesses, the FDA uses special procedures designed "to expedite the development, evaluation, and marketing of new therapies intended to treat persons with life-threatening and severely-debilitating illnesses, especially where no satisfactory alternative therapy exists." 21 C.F.R. § 312.80. [2]

In reviewing these types of drugs, the FDA recognizes the need for "a medical risk-benefit judgment" and therefore "will consider whether the benefits of the drug outweigh the known and potential risks of the drug and the need to answer remaining questions about risks and benefits of the drug, taking into consideration the severity of the disease and the absence of satisfactory alternative therapy." 21 C.F.R. §312.84(a).

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[1] "How FDA Evaluates Regulated Products: Drugs" (http://www.fda.gov/AboutFDA/Transparency/Basics/ucm269834.htm).

[2] The FDA has explained the reason for utilizing these special procedures as follows:

> As stated § 314.105(c) of this chapter, while the statutory standards of safety and effectiveness apply to all drugs, the many kinds of drugs that are subject to them, and the wide range of uses for those drugs, demand flexibility in applying the standards. The Food and Drug Administration (FDA) has determined that it is appropriate to exercise the broadest flexibility in applying the statutory standards, while preserving appropriate guarantees for safety and effectiveness. These procedures reflect the recognition that physicians and patients are generally willing to accept greater risks or side effects from products that treat life-threatening and severely-debilitating illnesses, than they would accept from products that treat less serious illnesses. These procedures also reflect the recognition that the benefits of the drug need to be evaluated in light of the severity of the disease being treated. The procedure outlined in this section should be interpreted consistent with that purpose.

21 C.F.R. § 312.80.